

CIRM 2.0 Kicks Off With Investments in Promising Therapies for Deadly Form of Skin Cancer and Vision Destroying Disease

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San Francisco, CA – A Phase 3 clinical trial for advanced melanoma and essential work that could lead to a clinical trial for a therapy for retinitis pigmentosa are the first two projects under CIRM 2.0 to get approval from the governing Board of CIRM, California's stem cell agency.

The Board awarded NeoStem \$17.7 million to carry out a Phase 3 clinical trial targeting cancer stem cells. These cells are believed to be able to survive chemotherapy and other cancer-targeting treatments, and can cause a relapse by enabling tumors to grow and spread. This approach uses the patient's own tumor cells to create a personalized therapy, one designed to engage the patient's own immune system and destroy the cancer.

In 2014 there were approximately 20,000 new cases of metastatic melanoma and nearly 10,000 deaths. It usually causes death within one to two years and only 10 to 15 percent of patients survive five years.

The NeoStem award is the first time CIRM has funded a Phase 3 clinical trial. This double-blind study will enroll approximately 250 patients at more than 40 sites worldwide, including 7 in California. Previous studies show that the therapy is well tolerated by patients and, unlike existing methods, does not appear to have severe or toxic side effects.

The Board also awarded \$4.95 million to Cedars-Sinai to develop a therapy for retinitis pigmentosa, a disease that often starts early in life and slowly destroys a person's vision. The award will fund the late-stage research needed to apply to the Food and Drug Administration (FDA) for approval for a clinical trial in people. The therapy will involve injecting neural progenitor cells under the retina at the back of the eye. The hope is that this will slow or even halt the progress of the disease.

"CIRM 2.0 is designed to accelerate the development of treatments for people with unmet medical needs, and these two projects clearly fit that description," says Dr. C. Randal Mills, President and CEO of CIRM. "With the Board's approval today we will now get this work up and running within the next 45 days. But that's just the start. We are not just providing financial support, we are also partnering with these groups to provide expertise, guidance and other kinds of support that these teams need to help them be successful. That's the promise of CIRM 2.0. Faster funding, better programs and a more comprehensive approach to supporting their progress."

The meeting also featured the swearing-in of two new Board members. Adriana Padilla, M.D. and Robert Price, Ph.D.

Dr. Padilla is a family physician, a member of the University of California, San Francisco-Fresno medical faculty, and an award-winning researcher with expertise in diabetes and its impact on Latino families and the health system in California's Central Valley. She is also active in the National Hispanic Medical Association (NHMA) and is also a member of the American Diabetes Association.

Dr. Price is the Associate Vice Chancellor for Research and a Professor of Political Science at U.C. Berkeley. His academic and teaching interests include comparative politics, with a particular interest in the politics of South Africa. This is Dr. Price's second time on the Board. He previously served as the alternate to UC Berkeley Chancellor Robert Birgeneau.

Both were appointed to the Board by Lt. Governor Gavin Newsom.

"We are delighted to welcome Adriana to the Board and welcome Bob back," says Jonathan Thomas, Ph.D., J.D., Chair of the Board. "They come from very different backgrounds and with very different areas of expertise, but both will be a great addition to the Board. They have both dedicated themselves to helping the people of California and we look forward to benefitting from their leadership and patient advocacy."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and to act with a sense of urgency commensurate with that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to r

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